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(54) Title: METHODS AND COMPOSITIONS FOR REGULATION AND MANIPULATION OF STEROIDOGENESIS

(57) Abstract: The steroidogenic acute regulatory protein (StAR), a mitochondrial protein required for stress responses, reproduction, and sexual differentiation of male fetuses is shown to exert its activity transiently at the outer mitochondrial membrane (OMM) rather than in its final resting place in the matrix, and that its OMM residency time and activity are regulated by its speed of mitochondrial import. This may be the first example of a mitochondrial protein exerting its biological activity in a compartment other than that to which it is finally targeted. This unique system which permits steroidogenic cells to initiate and terminate massive levels of steroidogenesis within a few minutes, permitting the rapid regulation of serum steroid hormone concentrations therefore can be manipulated by altering the binding of the leader sequence for the StAR protein to its receptor on the OMM.



## INTERNATIONAL SEARCH REPORT

International application No.

PCT/US03/15906

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| B. FIELDS SEARCHED   |  |   |   |  |
| Minimum documentation searched (classification system followed by classification symbols) U.S.: 435/6; 536/23.1, 23.4, 23.5, 24.1, 24.2  |  |   |   |  |
| Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched  |  |   |   |  |
| Electronic data base consulted during the international search (name of data base and, where practicable, search terms used) STN AND WEST.   |  |   |   |  |
| C. DOCUMENTS CONSIDERED TO BE RELEVANT   |  |   |   |  |
| Category *   | Citation of document, with indication, where a   | propriate,  | , of the relevant passages Relevant to claim No.  |  |
| A  | US 6,194,555 B1 (STOCCO et al.) 27 February 20   | 01 (27.02.2   | 2001), see the entire patent.   |  |
| A  | US 5,872,230 A (STOCCO et al.) 16 February 199   | 9 (16.02.19   | 1-18  |  |
|  |  |   |   |  |
|  | r documents are listed in the continuation of Box C.   | -T"   | See patent family annex.  later document published after the international filing date or priority  |  |
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